General

Guideline Title

Evidence-based guideline summary: diagnosis and treatment of limb-girdle and distal dystrophies: report of the Guideline Development Subcommittee of the American Academy of Neurology and the Practice Issues Review Panel of the American Association of Neuromuscular and Electrodiagnostic Medicine.

Bibliographic Source(s)

Narayanaswami P, Weiss M, Selcen D, David W, Raynor E, Carter G, Wicklund M, Barohn RJ, Ensrud E, Griggs RC, Gronseth G, Amato AA. Evidence-based guideline summary: diagnosis and treatment of limb-girdle and distal dystrophies: report of the Guideline Development Subcommittee of the American Academy of Neurology and the Practice Issues Review Panel of the American Association of Neuromuscular and Electrodiagnostic Medicine. Neurology. 2014 Oct 14;83(16):1453-63. [40 references] PubMed

Guideline Status

This is the current release of the guideline.

This guideline meets NGC's 2013 (revised) inclusion criteria.

Recommendations

Major Recommendations

Definitions of the levels of the recommendations (A, B, C, U, R) and classification of the evidence (Class I-IV) are provided at the end of the "Major Recommendations" field.

Note from the National Guideline Clearinghouse (NGC): Refer to the LGMD Guideline (see the "Availability of Companion Documents" field) for the clinical context of the recommendations.

Major Practice Recommendations

The recommendations below encompass three major areas: diagnosis, evaluation, and management of muscular dystrophies. The full recommendation set is available online.

Diagnosis of Muscular Dystrophies

For patients with suspected muscular dystrophy, clinicians should use a clinical approach to guide genetic diagnosis based on the clinical phenotype, including the pattern of muscle involvement, inheritance pattern, age at onset, and associated manifestations (e.g., early contractures, cardiac or respiratory involvement) (Level B).

In patients with suspected muscular dystrophy in whom initial clinically directed genetic testing does not provide a diagnosis, clinicians may obtain genetic consultation or perform parallel sequencing of targeted exomes, whole-exome sequencing, whole-genome screening, or next-generation sequencing to identify the genetic abnormality (Level C).

Evaluation and Medical Management of Muscular Dystrophies

Cardiac Involvement

Clinicians should refer newly diagnosed patients with (1) limb-girdle muscular dystrophy (LGMD)1A, LGMD1B, LGMD1D, LGMD1E, LGMD2C–K, LGMD2M–P, Becker muscular dystrophy (BMD), Emery-Dreifuss muscular dystrophy (EDMD), and myofibrillar myopathies (MFM) or (2) muscular dystrophy without a specific genetic diagnosis for cardiology evaluation, including electrocardiogram (ECG) and structural evaluation (echocardiography or cardiac magnetic resonance imaging [MRI]), even if they are asymptomatic from a cardiac standpoint, to guide appropriate management (Level B).

If ECG or structural cardiac evaluation (e.g., echocardiography) has abnormal results, or if the patient has episodes of syncope, near-syncope, or palpitations, clinicians should order rhythm evaluation (e.g., Holter monitor or event monitor) to guide appropriate management (Level B).

Clinicians should refer muscular dystrophy patients with palpitations, symptomatic or asymptomatic tachycardia or arrhythmias, or signs and symptoms of cardiac failure for cardiology evaluation (Level B).

It is not obligatory for clinicians to refer patients with LGMD2A, LGMD2B, and LGMD2L for cardiac evaluation unless they develop overt cardiac signs or symptoms (Level B).

Dysphagia and Nutrition

Clinicians should refer muscular dystrophy patients with dysphagia, frequent aspiration, or weight loss for swallowing evaluation or gastroenterology evaluation to assess and manage swallowing function and aspiration risk, to teach patients techniques for safe and effective swallowing (e.g., chin tuck maneuver, altered food consistencies), and to consider placement of a gastrostomy/jejunostomy tube for nutritional support (Level B).

Pulmonary Complications

Clinicians should order pulmonary function testing (spirometry and maximal inspiratory/expiratory force in the upright and, if normal, supine positions) or refer for pulmonary evaluation (to identify and treat respiratory insufficiency) in muscular dystrophy patients at the time of diagnosis, or if they develop pulmonary symptoms later in their course (Level B).

In patients with a known high risk of respiratory failure (e.g., those with LGMD2I or MFM), clinicians should obtain periodic pulmonary function testing (spirometry and maximal inspiratory/expiratory force in the upright position and, if normal, in the supine position) or evaluation by a pulmonologist to identify and treat respiratory insufficiency (Level B).

It is not obligatory for clinicians to refer patients with LGMD2B and LGMD2L for pulmonary evaluation unless they are symptomatic (Level C).

Clinicians should refer muscular dystrophy patients with excessive daytime somnolence, nonrestorative sleep (e.g., frequent nocturnal arousals, morning headaches, excessive daytime fatigue), or respiratory insufficiency based on pulmonary function tests for pulmonary or sleep medicine consultation for consideration of noninvasive ventilation to improve quality of life (Level B).

Spinal Deformities

Clinicians should monitor patients with muscular dystrophy for the development of spinal deformities to prevent resultant complications and preserve function (Level B).

Clinicians should refer muscular dystrophy patients with musculoskeletal spine deformities to an orthopedic spine surgeon for monitoring and surgical intervention if it is deemed necessary in order to maintain normal posture, assist mobility, maintain cardiopulmonary function, and optimize quality of life (Level B).

Rehabilitative Management and Treatment of Muscular Dystrophies

Clinical Rehabilitative Management

Clinicians should refer patients with muscular dystrophy to a clinic that has access to multiple specialties (e.g., physical therapy, occupational therapy, respiratory therapy, speech and swallowing therapy, cardiology, pulmonology, orthopedics, and genetics) designed specifically to care for

patients with muscular dystrophy and other neuromuscular disorders in order to provide efficient and effective long-term care (Level B).

Clinicians should recommend that patients with muscular dystrophy have periodic assessments by a physical and occupational therapist for symptomatic and preventive screening (Level B).

While respecting and protecting patient autonomy, clinicians should proactively anticipate and facilitate patient and family decision-making as the disease progresses, including decisions regarding loss of mobility, need for assistance with activities of daily living, medical complications, and end-of-life care (Level B).

For patients with muscular dystrophy, clinicians should prescribe physical and occupational therapy, as well as bracing and assistive devices that are adapted specifically to the patient's deficiencies and contractures, in order to preserve mobility and function and prevent contractures (Level B).

Strength Training and Aerobic Exercise Training

Clinicians may advise patients with muscular dystrophy that aerobic exercise combined with a supervised submaximal strength training program is probably safe (Level C).

Clinicians may advise patients with muscular dystrophy that gentle, low-impact aerobic exercise (swimming, stationary bicycling) improves cardiovascular performance, increases muscle efficiency, and lessens fatigue (Level C).

Clinicians may counsel patients with muscular dystrophy to hydrate adequately, not to exercise to exhaustion, and to avoid supramaximal, high-intensity exercise (Level C).

Clinicians should educate patients with muscular dystrophy who are participating in an exercise program about the warning signs of overwork weakness and myoglobinuria, which include feeling weaker rather than stronger within 30 minutes after exercise, excessive muscle soreness 24–48 hours following exercise, severe muscle cramping, heaviness in the extremities, and prolonged shortness of breath (Level B).

Medical Treatments

Clinicians should not currently offer patients with muscular dystrophy gene therapy, myoblast transplantation, neutralizing antibody to myostatin, or growth hormone outside of a research study designed to determine the efficacy and safety of the treatment (Level R).

Definitions:

Classification of Evidence Schemes

Screening

Class I: A statistical, population-based sample of patients studied at a uniform point in time (usually early) during the course of the condition. All patients undergo the intervention of interest. The outcome, if not objective, is determined in an evaluation that is masked to the patients' clinical presentations.

Class II: A statistical, non-referral-clinic-based sample of patients studied at a uniform point in time (usually early) during the course of the condition. Most patients undergo the intervention of interest. The outcome, if not objective, is determined in an evaluation that is masked to the patients' clinical presentations.

Class III: A sample of patients studied during the course of the condition. Some patients undergo the intervention of interest. The outcome, if not objective, is determined in an evaluation by someone other than the treating physician.

Class IV: Studies not meeting Class I, II or III criteria including consensus, expert opinion or a case report

Therapy

Class I: Prospective, randomized, controlled clinical trial with masked outcome assessment, in a representative population. The following are required:

a. Concealed allocation
b. Primary outcome(s) clearly defined
c. Exclusion/inclusion criteria clearly defined
d. Adequate accounting for drop-outs and cross-overs with numbers sufficiently low to have minimal potential for bias
e. Relevant baseline characteristics are presented and substantially equivalent among treatment groups or there is appropriate statistical

adjustment for differences

Class II: Prospective matched group cohort study in a representative population with masked outcome assessment that meets a-e above OR a RCT in a representative population that lacks one criteria a-d.

Class III: All other controlled trials (including well-defined natural history controls or patients serving as own controls) in a representative population, where outcome is independently assessed, or independently derived by objective outcome measurement.

Class IV: Evidence from uncontrolled studies, case series, case reports, or expert opinion.

Classification of Recommendations

Level A = Established as effective, ineffective or harmful (or established as useful/predictive or not useful/predictive) for the given condition in the specified population. (Level A rating requires at least two consistent Class I studies.)*

Level B = Probably effective, ineffective or harmful (or probably useful/predictive or not useful/predictive) for the given condition in the specified population. (Level B rating requires at least one Class I study or two consistent Class II studies.)

Level C = Possibly effective, ineffective or harmful (or possibly useful/predictive or not useful/predictive) for the given condition in the specified population. (Level C rating requires at least one Class II study or two consistent Class III studies.)

Level U = Data inadequate or conflicting; given current knowledge, treatment (test, predictor) is unproven.

Level R = Designates that the intervention should not be used outside of a research setting.

*In exceptional cases, one convincing Class I study may suffice for an "A" recommendation if 1) all criteria are met, 2) the magnitude of effect is large (relative rate improved outcome >5 and the lower limit of the confidence interval is >2).

Clinical Algorithm(s)

The following algorithms are provided in the original guideline document:

- Conceptual approach to a patient with a suspected limb-girdle muscular dystrophy
- Diagnostic approach to patients with a limb-girdle pattern of weakness and suspected muscular dystrophy with an autosomal dominant inheritance pattern
- Diagnostic approach to patients with a limb-girdle pattern of weakness and suspected muscular dystrophy with an autosomal recessive inheritance pattern
- Diagnostic approach to patients with a limb-girdle pattern of weakness and suspected muscular dystrophy with an X-linked recessive inheritance pattern

The following algorithms are provided in the data supplement:

- Diagnostic approach to patients with a humeroperoneal pattern of weakness and suspected muscular dystrophy (Emery-Dreifuss muscular dystrophy)
- Diagnostic approach to patients with a distal pattern of weakness and suspected muscular dystrophy

Scope

Disease/Condition(s)

Limb-girdle muscular dystrophies (LGMDs) and distal dystrophies, including:

- Hereditary myopathies (e.g., hereditary inclusion body myopathies [hIBMs], Emery-Dreifuss muscular dystrophy [EDMD], Becker muscular dystrophy [BMD])
- Non-limb-girdle adult-onset myopathies (e.g., Miyoshi distal myopathy, myofibrillar myopathies [MFM])

Note: Duchenne dystrophy, congenital muscular dystrophy, myotonic dystrophy, and facioscapulohumeral dystrophy are not included in this

guideline, as they will be discussed in forthcoming guidelines. The terms LGMD and muscular dystrophy are used interchangeably to refer to the disorders reviewed in this guideline.

Guideline Category

Diagnosis

Evaluation

Management

Treatment

Clinical Specialty

Cardiology

Neurology

Pulmonary Medicine

Intended Users

Physician Assistants

Physicians

Respiratory Care Practitioners

Guideline Objective(s)

To review the current evidence and make practice recommendations regarding the diagnosis and treatment of limb-girdle muscular dystrophies (LGMDs)

Target Population

Patients with or suspected of having limb-girdle muscular dystrophies (LGMDs)

Interventions and Practices Considered

Diagnosis/Evaluation

1. Clinical approach to diagnosis based on clinical phenotype
    - Genetic consultation and testing
    - Parallel sequencing of target exomes
2. Referral for cardiology evaluation and rhythm evaluation (if indicated)
3. Referral for swallowing evaluation (dysphagia)
4. Referral for pulmonary function tests or sleep medicine consultation for consideration of noninvasive ventilation (excessive daytime somnolence, nonrestorative sleep)
5. Monitoring patients for spinal deformities and referral to orthopedic spine surgeon if indicated

Management/Treatment

1. Referral to multiple specialties clinic (e.g., physical therapy, occupational therapy, respiratory therapy, speech and swallowing therapy,

cardiology, pulmonology, orthopedics, and genetics)
    - Inclusion of periodic assessments
    - Facilitation of patient and family decision-making
    - Prescription for physical and occupational therapy, bracing and assistive devices
2. Exercise program
    - Low-impact aerobics and submaximal strength training
    - Proper hydration
    - Awareness of overwork weakness and myoglobinuria

Note: The following interventions were considered but not recommended: muscular dystrophy gene therapy, myoblast transplantation, neutralizing antibody to myostatin, or growth hormone outside of a research study designed to determine the efficacy and safety of the treatment.

Major Outcomes Considered

- Efficacy and safety of the treatments
- Cardiac complications
- Respiratory failure
- Maintaining adequate nutrition and body weight
- Quality of life

Methodology

Methods Used to Collect/Select the Evidence

Searches of Electronic Databases

Description of Methods Used to Collect/Select the Evidence

In July 2010, the American Academy of Neurology (AAN) Guideline Development Subcommittee and the American Association of Neuromuscular and Electrodiagnostic Medicine Practice Issues Review Panel (see Appendices e-1-e-3 in the LGMD Guideline [see the "Availability of Companion Documents" field]) formed a panel of neurologists, other physicians with relevant expertise, methodologists, and patient advocates. The MEDLINE, EMBASE, and Cochrane databases were searched from 1987 onward for relevant peer-reviewed articles in humans and in English only (Appendix e-4 in the LGMD Guideline provides the full search strategy and terms). Through an initial search conducted in 2011 and an updated search conducted in 2013, a total of 3,246 abstracts were identified. Of those, 1,335 articles were selected for full-text review. Two panel members, working independently of each other, reviewed each of the 1,335 articles and selected 699 for final review and classification.

Number of Source Documents

699 were selected for final review and classification

Methods Used to Assess the Quality and Strength of the Evidence

Weighting According to a Rating Scheme (Scheme Given)

Rating Scheme for the Strength of the Evidence

Classification of Evidence Schemes

Screening

Class I: A statistical, population-based sample of patients studied at a uniform point in time (usually early) during the course of the condition. All

patients undergo the intervention of interest. The outcome, if not objective, is determined in an evaluation that is masked to the patients' clinical presentations.

Class II: A statistical, non-referral-clinic-based sample of patients studied at a uniform point in time (usually early) during the course of the condition. Most patients undergo the intervention of interest. The outcome, if not objective, is determined in an evaluation that is masked to the patients' clinical presentations.

Class III: A sample of patients studied during the course of the condition. Some patients undergo the intervention of interest. The outcome, if not objective, is determined in an evaluation by someone other than the treating physician.

Class IV: Studies not meeting Class I, II or III criteria including consensus, expert opinion or a case report

Therapy

Class I: Prospective, randomized, controlled clinical trial with masked outcome assessment, in a representative population. The following are required:

a. Concealed allocation
b. Primary outcome(s) clearly defined
c. Exclusion/inclusion criteria clearly defined
d. Adequate accounting for drop-outs and cross-overs with numbers sufficiently low to have minimal potential for bias
e. Relevant baseline characteristics are presented and substantially equivalent among treatment groups or there is appropriate statistical adjustment for differences

Class II: Prospective matched group cohort study in a representative population with masked outcome assessment that meets a-e above OR a RCT in a representative population that lacks one criteria a-d.

Class III: All other controlled trials (including well-defined natural history controls or patients serving as own controls) in a representative population, where outcome is independently assessed, or independently derived by objective outcome measurement.

Class IV: Evidence from uncontrolled studies, case series, case reports, or expert opinion.

Methods Used to Analyze the Evidence

Systematic Review

Description of the Methods Used to Analyze the Evidence

Each final article was reviewed by 2 panel members who rated it according to the American Academy of Neurology (AAN) 2011 criteria for classification of articles (see Appendix e-5 in the LGMD Guideline [see the "Availability of Companion Documents" field]), using the scheme appropriate to the clinical question. The AAN population screening evidence scheme was used for questions 1–4, and the therapeutic scheme for question 5. Where differences in article ratings occurred, a third panel member determined the ultimate rating.

Methods Used to Formulate the Recommendations

Expert Consensus (Delphi)

Description of Methods Used to Formulate the Recommendations

The systematic review and practice recommendations were developed according to the processes described in the 2004 and 2011 American Academy of Neurology (AAN) guideline development process manuals. Appendices e-1 through e-6 in the LGMD Guideline (see "Availability of Companion Documents" field) provide further background on development of this guideline.

To inform recommendations regarding the diagnosis, management, and treatment of limb-girdle muscular dystrophies (LGMDs), the authors performed systematic reviews to answer the following questions:

1. What is the frequency of genetically confirmed LGMD subtypes?
2. How often do patients with muscular dystrophy and its specific subtypes have specific clinical features, including ethnic predilection, diagnostic patterns of weakness, respiratory and cardiac complications, laboratory abnormalities (e.g., elevated creatine kinase [CK]), specific patterns on imaging, and muscle biopsy features?
3. Are there effective therapies for muscular dystrophies?

The conclusions from the systematic reviews informed the practice recommendation development process. The author panel developed actionable practice recommendations following an explicit structured process. Levels of obligation associated with each recommendation were assigned using a modified Delphi process.

Rating Scheme for the Strength of the Recommendations

Classification of Recommendations

Level A = Established as effective, ineffective or harmful (or established as useful/predictive or not useful/predictive) for the given condition in the specified population. (Level A rating requires at least two consistent Class I studies.)*

Level B = Probably effective, ineffective or harmful (or probably useful/predictive or not useful/predictive) for the given condition in the specified population. (Level B rating requires at least one Class I study or two consistent Class II studies.)

Level C = Possibly effective, ineffective or harmful (or possibly useful/predictive or not useful/predictive) for the given condition in the specified population. (Level C rating requires at least one Class II study or two consistent Class III studies.)

Level U = Data inadequate or conflicting; given current knowledge, treatment (test, predictor) is unproven.

Level R = Designates that the intervention should not be used outside of a research setting.

*In exceptional cases, one convincing Class I study may suffice for an "A" recommendation if 1) all criteria are met, 2) the magnitude of effect is large (relative rate improved outcome >5 and the lower limit of the confidence interval is >2).

Cost Analysis

A formal cost analysis was not performed and published cost analyses were not reviewed.

Method of Guideline Validation

External Peer Review

Internal Peer Review

Description of Method of Guideline Validation

Drafts of the guideline have been reviewed by at least three American Academy of Neurology (AAN) committees, at least one American Association of Neuromuscular and Electrodiagnostic Medicine (AANEM) committee, a network of neurologists, Neurology peer reviewers, and representatives from related fields.

This guideline was approved by the AAN Guideline Development Subcommittee on July 13, 2013; by the AAN Practice Committee on February 3, 2014; by the AANEM Board of Directors on July 10, 2014; and by the AANI Board of Directors on July 7, 2014.

This guideline was endorsed by the American Academy of Physical Medicine and Rehabilitation on April 17, 2014; by the Child Neurology Society on July 11, 2014; by the Jain Foundation on March 14, 2013; and by the Muscular Dystrophy Association on August 27, 2014.

Evidence Supporting the Recommendations

Type of Evidence Supporting the Recommendations

The type of supporting evidence is identified and graded for each recommendation (see the "Major Recommendations" field).

Benefits/Harms of Implementing the Guideline Recommendations

Potential Benefits

Appropriate diagnosis and treatment of limb-girdle and distal dystrophies

Potential Harms

With strengthening and aerobic fitness training programs, due to the muscle degeneration in muscular dystrophy, there may be some risk of exercise-induced muscle damage, myoglobinuria, and subsequent overwork weakness following supramaximal, high-intensity exercise.

Qualifying Statements

Qualifying Statements

This statement is provided as an educational service of the American Academy of Neurology (AAN) and American Association of Neuromuscular and Electrodiagnostic Medicine (AANEM). It is based on an assessment of current scientific and clinical information. It is not intended to include all possible proper methods of care for a particular neurologic problem or all legitimate criteria for choosing to use a specific procedure. Neither is it intended to exclude any reasonable alternative methodologies. The AAN and AANEM recognize that specific patient care decisions are the prerogative of the patient and the physician caring for the patient, based on all of the circumstances involved. The clinical context section is made available in order to place the evidence-based guidelines into perspective with current practice habits and challenges. Formal practice recommendations are not intended to replace clinical judgment.

Implementation of the Guideline

Description of Implementation Strategy

An implementation strategy was not provided.

Implementation Tools

Clinical Algorithm

Patient Resources

Quick Reference Guides/Physician Guides

Slide Presentation

Staff Training/Competency Material

For information about availability, see the *Availability of Companion Documents* and *Patient Resources* fields below.

Institute of Medicine (IOM) National Healthcare Quality Report

Categories

IOM Care Need

Getting Better

Living with Illness

IOM Domain

Effectiveness

Patient-centeredness

Identifying Information and Availability

Bibliographic Source(s)

Narayanaswami P, Weiss M, Selcen D, David W, Raynor E, Carter G, Wicklund M, Barohn RJ, Ensrud E, Griggs RC, Gronseth G, Amato AA. Evidence-based guideline summary: diagnosis and treatment of limb-girdle and distal dystrophies: report of the Guideline Development Subcommittee of the American Academy of Neurology and the Practice Issues Review Panel of the American Association of Neuromuscular and Electrodiagnostic Medicine. Neurology. 2014 Oct 14;83(16):1453-63. [40 references] PubMed

Adaptation

Not applicable: The guideline was not adapted from another source.

Date Released

2014 Oct 14

Guideline Developer(s)

American Academy of Neurology - Medical Specialty Society

American Association of Neuromuscular and Electrodiagnostic Medicine - Medical Specialty Society

Source(s) of Funding

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Guideline Committee

Guideline Development Subcommittee of the American Academy of Neurology and the Practice Issues Review Panel of the American Association of Neuromuscular and Electrodiagnostic Medicine

Composition of Group That Authored the Guideline

Guideline Authors: Pushpa Narayanaswami, MBBS, DM, FAAN; Michael Weiss, MD, FAAN; Duygu Selcen, MD; William David, MD, PhD; Elizabeth Raynor, MD; Gregory Carter, MD; Matthew Wicklund, MD, FAAN; Richard J. Barohn, MD, FAAN; Erik Ensrud, MD; Robert C. Griggs, MD, FAAN; Gary Gronseth, MD, FAAN; Anthony A. Amato, MD, FAAN

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Financial Disclosures/Conflicts of Interest

Conflict of Interest

The American Academy of Neurology (AAN) and American Association of Neuromuscular and Electrodiagnostic Medicine (AANEM) are committed to producing independent, critical, and truthful clinical practice guidelines (CPGs). Significant efforts are made to minimize the potential for conflicts of interest to influence the recommendations of this CPG. To the extent possible, the AAN and AANEM keep separate those who have a financial stake in the success or failure of the products appraised in the CPGs and the developers of the guidelines. Conflict of interest forms were obtained from all authors and reviewed by an oversight committee prior to project initiation. AAN and AANEM limit the participation of authors with substantial conflicts of interest. The AAN and AANEM forbid commercial participation in, or funding of, guideline projects. Drafts of the guideline have been reviewed by at least 3 AAN committees, at least 1 AANEM committee, a network of neurologists, Neurology peer reviewers, and representatives from related fields. The AAN Guideline Author Conflict of Interest Policy can be viewed at www.aan.com. For complete information on this process, access the 2004 AAN process manual.

Disclosures

P. Narayanaswami has received honoraria from the AAN and the AANEM.

M. Weiss has served as a speaker for the AAN, AANEM, American Academy of Physical Medicine & Rehabilitation (AAPM&R), Athena Diagnostics, Nufactor, Walgreens, and Grifols Inc.; serves on speakers' bureaus for Athena Diagnostics and Walgreens; has consulted for Genzyme Corporation, CSL Behring, Questcor Pharmaceuticals, and Washington State Labor and Industries; and has received research funding support from the ALS Therapy Alliance and Northeast ALS Consortium.

D. Selcen has served as an editorial board member for *Neuromuscular Disorders* and has received funding for research from the National Institutes of Health (NIH).

W. David and E. Raynor report no disclosures relevant to the manuscript.

G. Carter has served as the senior associate editor for *Muscle & Nerve*, has received honoraria from the AANEM and the Canadian Association of Physical Medicine and Rehabilitation, has received funding for research from the National Institutes on Aging and the National Institute on Disability and Rehabilitation Research, and has testified on a case regarding the use of marijuana in pain.

M. Wicklund has served on a scientific advisory board for Sarepta Therapeutics, has served on a speakers' bureau for Genzyme, has received grant funding from Eli Lilly, and has collaborated on research without compensation with Athena Diagnostics.

R. Barohn has served as a consultant or on a scientific advisory board for Genzyme, Grifols, MedImmune, and Novartis; has received honoraria from Alexion, Isis, Baxter, Sarepta, and CSL Behring; and has received funding for research from the U.S. Food and Drug Administration (FDA) and the NIH.

E. Ensrud reports no relevant disclosures.

R. Griggs consults for PTC Therapeutics (Chair of DSMB), Novartis (DSMB), Marathon Pharmaceuticals, Taro Pharmaceuticals, and Viromed (DSMB); receives funding from the NIH, the Italian Telethon (DSMB Chair), the Muscular Dystrophy Association, the Parent Project for Muscular Dystrophy, and the AAN; and receives royalties from Elsevier (for *Cecil Essentials* and *Cecil Textbook of Medicine*).

G. Gronseth serves as an editorial advisory board member of *Neurology Now*, is an associate editor of *Neurology*, and receives honoraria from

the AAN.

A. Amato has served as a consultant or on scientific advisory boards for MedImmune, Amgen, Biogen, DART, and Baxter; serves as an associate editor for *Neurology* and *Muscle & Nerve*; has received royalties from publishing from *Neuromuscular Disorders*; has received honoraria from the AAN and AANEM; and has received funding for research from Amgen, MedImmune, Novartis, the FDA, and the NIH.

Go to Neurology.org for full disclosures.

Guideline Endorser(s)

American Academy of Physical Medicine and Rehabilitation - Medical Specialty Society

Child Neurology Society - Medical Specialty Society

Jain Foundation - Disease Specific Society

Muscular Dystrophy Association - Disease Specific Society

Guideline Status

This is the current release of the guideline.

This guideline meets NGC's 2013 (revised) inclusion criteria.

Guideline Availability

Electronic copies: A list of American Academy of Neurology (AAN) guidelines, along with a link to this guideline, is available from the AAN Web site.

Print copies: Available from the AAN Member Services Center, (800) 879-1960, or from AAN, 201 Chicago Avenue South, Minneapolis, MN 55415.

Availability of Companion Documents

The following are available:

- Evidence-based guideline summary: diagnosis and treatment of limb-girdle and distal dystrophies. Data supplement (LGMD Guideline, e-figures, e-references, e-tables). St. Paul (MN): American Academy of Neurology; 2014. Electronic copies: Available from the Neurology Journal Web site.
- Evidence-based guideline: diagnosis and treatment of limb-girdle and distal muscular dystrophies. Summary of evidence-based guideline for clinicians. St. Paul (MN): American Academy of Neurology. 2014. 17 p. Electronic copies: Available from the American Academy of Neurology (AAN) Web site.
- Diagnosis and treatment of limb-girdle and distal dystrophies. Case presentation. St. Paul (MN): American Academy of Neurology. 2014. 9 p. Electronic copies: Available from the AAN Web site.
- Evidence-based guideline summary: diagnosis and treatment of limb-girdle and distal dystrophies. Slide presentation. St. Paul (MN): American Academy of Neurology. 2014. Electronic copies: Available from the AAN Web site.
- AAN guideline development process [online]. St. Paul (MN): American Academy of Neurology. Electronic copies: Available from the AAN Web site.

Patient Resources

The following is available:

- Limb-girdle and distal muscular dystrophies. Summary of evidence-based guideline for patients and their families. St. Paul (MN): American

Academy of Neurology; 2014. 4 p. Electronic copies: Available from the American Academy of Neurology (AAN) Web site .

Please note: This patient information is intended to provide health professionals with information to share with their patients to help them better understand their health and their diagnosed disorders. By providing access to this patient information, it is not the intention of NGC to provide specific medical advice for particular patients. Rather we urge patients and their representatives to review this material and then to consult with a licensed health professional for evaluation of treatment options suitable for them as well as for diagnosis and answers to their personal medical questions. This patient information has been derived and prepared from a guideline for health care professionals included on NGC by the authors or publishers of that original guideline. The patient information is not reviewed by NGC to establish whether or not it accurately reflects the original guideline's content.

NGC Status

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